

Title (en)
LYSOSOMAL TARGETING PATHWAY ENZYMES

Title (de)
ENZYME DES AUF DIE LYSOSOMEN GERICHTETEN WEGES

Title (fr)
ENZYMES A VOIE DE CIBLAGE LYSOSOMIAL

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Application
EP 00961335 A 20000914

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Abstract (en)
[origin: WO0119955A2] The lysosomal targeting pathway enzymes GlcNAc-phosphotransferase and phosphodiester alpha -GlcNAcase are recovered and used to produce highly phosphorylated lysosomal hydrolases that can be used to treat lysosomal storage diseases. Generally, the nucleic acid molecules coding for the enzymes are incorporated into expression vectors that are used to transfect host cells that express the enzymes. The expressed enzymes are recovered and used to treat lysosomal hydrolases having high mannose structures. The resulting lysosomal hydrolases have asparagine-linked oligosaccharides that are highly phosphorylated with mannose phosphate. The highly phosphorylated lysosomal hydrolases are readily taken into the cell and into the lysosome during enzyme replacement therapy procedures.

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